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The national spina bifida patient registry past, present, and future

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Abstract

"The National Spina Bifida Patient Registry: Past, Present, and Future" was presented at the Spina Bifida World Congress, March 17, 2017, San Diego, California. This commentary provides a summary of registry activities including the reason for development, a description of the clinic participants and their patients who are participating, analytic works and publications. Two specific efforts that are related to the work of the registry, a urologic protocol to preserve renal function for newborns and young children, and a skin breakdown prevention bundle developed and implemented in registry clinics, are highlighted.

Keywords

Spina bifida; patient registry; interventions; outcomes

1. Introduction

It is my pleasure to describe the work at the Centers for Disease Control and Prevention (CDC) to improve health outcomes and quality of life among people living with spina bifida. I do this on behalf of my colleagues on the Rare Disorders and Health Outcomes Team in the National Center on Birth Defects and Developmental Disabilities, CDC, and the healthcare and research professionals who collaborate with us in development, enhancements and data analysis from participating spina bifida clinics.

My comments will address the origin and purpose of the National Spina Bifida Patient Registry (NSBPR), objectives, what we have learned thus far, current activities, and plans for the future.

2. Background

In 2003, CDC, the Spina Bifida Association (SBA), the Agency for Healthcare Research and Quality (AHRQ), and the National Institutes of Health (NIH) sponsored a conference entitled "Evidence-Based Practice in Spina Bifida: Developing a Research Agenda" after which the proceedings were published in booklet form by Liptak (2003) [1]. In 2006, an

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Conflict of interest

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assessment of spina bifida clinics in the United States conducted on behalf of the Spina Bifida Association by the Delmarva Foundation for Medical Care [Unpublished report], found variation in clinical services provided, and the impact of the care provided had not been evaluated. Staffing and services provided differed among clinics. Funded by congress, CDC collaborated with SBA to develop a clinical patient registry to accomplish three things: evaluate how practice affects patient outcomes; provide a foundation for clinical research; and improve health outcomes and quality of life for people affected by spina bifida.

With this charge, CDC has been collaborating with participating spina bifida (SB) clinics and receiving data describing clinical care provided and outcomes realized. Practices and outcomes can be compared among clinics so that best practices can be shared. Gaps in knowledge of care and outcomes can be identified using the registry and prioritized for future research, some of which can be accomplished using the aggregate registry data.

3. Implementation

Registry data collection began in 2009 when CDC funded 10 spina bifida clinics to establish the Registry and the data collection process. In 2011, 19 clinics participated to broaden and continue the collection of data to improve the care of people living with spina bifida. Today, 14 clinics are funded by CDC, and seven clinics are self-funded (Fig. 1). The focus in the current funding cycle, ending in 2019, is to implement research to describe best practices, compare differences in interventions and outcomes among clinics and to examine data longitudinally in children and adults.

Publications to date include a paper describing the methods to implement a registry to collect data [2]; a description of the initial registry participants [3]; an investigation of the association between sociodemographic characteristics and patient outcomes [4]; and identification of factors associated with mobility [5]. A description of the methods for developing and refining a urologic protocol for newborns and young children with spina bifida has also been published [6]. A paper titled, “Factors Associated with Pressure Ulcers in Individuals with Spina Bifida” [7] provides an example of how Registry data have been used to identify and promote improved care, and will be described in more detail below.

Figure 2 depicts how data flow from the clinics to a secure hosting site where data are encrypted and registry variables are parsed off and sent to CDC, as approved by each clinic’s Institutional Review Board. Aggregate data are compiled at the CDC and shared with the participating clinics who receive clinic-specific data reports as well.

4. Data

As of March 2017, the NSBPR includes 7,924 participants. Data collected include characteristics of the person with spina bifida and his/her family, such as grade in school, insurance, height and weight; the surgeries and procedures provided during the past year and throughout the life of the participant; results of urodynamics and sleep studies; bowel and bladder management techniques; and outcomes such as continence, mobility and skin breakdown.

The Registry participants range in age from birth to 89 years: 20% were older than 18 years at the time of enrollment, 22% were of Hispanic ethnicity and the most frequent diagnosis was myelomeningocele (78%), followed by lipomyelomeningocele (14%). Patients with the other eligible diagnoses, meningocele, split cord malformation, terminal myelocystocele, and fatty filum, contribute less than 6% of all cases.

Categorizing Registry participants into two groups, those diagnosed with myelomeningocele and those with other diagnoses, we found that

- 77% with myelomeningocele have a history of shunt placement
- among those older than five years in both groups, urinary continence is 40–52%, bowel continence 47–69%, and
- 4% had prenatal back closure.

Table 1 provides more descriptive information about registry participants.

At each clinic visit, registry participants are asked about their work/school status. Table 2 describes the vocational status of patients in the registry who are 22 years and older including those who are volunteering and those who are full time homemakers. Shunt placement is the most common surgery for patients with myelomeningocele and is included with other surgeries reported in Table 3. Surgeries for those with other eligible diagnoses are reported in Table 4.

Patient outcomes vary among clinics. Figure 3 exemplifies how outcomes data may vary among participating sites. In this figure, participating clinics are ranked by the prevalence of urinary continence among patients five years and older with a diagnosis of myelomeningocele. Examination of variation among clinic outcomes is expected to improve practices and processes as clinics with the lower proportions of favorable outcomes seek to emulate the practices of clinics with higher proportions of patients with favorable outcomes. We anticipate that providing these data to clinics will lead to identification and adoption of best practices, and ultimately, reduced variation in patient outcomes.

Analyses of Registry data in progress include description of treatments and interventions, which will highlight the associated challenges in decision-making; a comparison of variations among clinics in procedures patients have undergone; trends in outcomes over time; and bowel and bladder continence management techniques among adult patients.

5. Related projects

In order to address the question “what is the optimal bladder management protocol to preserve renal function in the newborn and young child with spina bifida?” CDC convened a group of urologic healthcare professionals, nephrologists, clinical epidemiologists, community advocates, and CDC personnel to develop a protocol to test for effectiveness.

Nine sites were funded in 2014 to prospectively treat all newborns with spina bifida using a single consensus-based protocol. Using the NSBPR, data collection began in February, 2015. To date, 153 infants have been enrolled, 79% of those eligible at participating clinics.

The urology protocol was developed using an iterative quality improvement design. Participating clinics commit to treat all newborns with a diagnosis of myelomeningocele using the protocol. The protocol specifies the timing of follow-up visits, the type and frequency of diagnostic procedures, and the type and nature of any treatment-related interventions. Care may be customized for particular patients, which results in explanation and documentation of the need for the customization. These protocol deviations are later analyzed to identify the need for potential modifications of the original protocol for either a subset of patients or the entire population.

The protocol dictates that the newborn will be catheterized for three days with decreasing frequency as long as the bladder volumes remain below 30 ml. The parents will be trained to do intermittent catheterization, which will be prescribed to continue at home only when bladder residuals are greater than 30 milliliters, or there is grade three or four hydronephrosis. Prophylactic antibiotics will not be prescribed unless there is Grade Five reflux, and a renal bladder ultrasound will be performed every three months in the first year. In years one through five, the protocol requires annual visits that include a renal bladder ultrasound and serum creatinine, with urodynamics through three years of age unless the bladder is characterized as hostile. In the fifth year, a measurement of the glomerular filtration rate is added.

Of particular interest are the occurrence and management of urinary tract infections (UTI) and the differentiation between lab confirmed infections and those diagnosed and treated based on symptoms alone. Various definitions of UTI were considered and the definition of Madden-Fuentes and colleagues was chosen for this study [8]. This urology protocol is the first iterative quality improvement consensus-based protocol to establish the most effective and efficient practice guidelines for the infant and young child affected by myelomeningocele.

Information from the NSBPR is also influencing clinical practice in the area of skin breakdown prevention. Analysis of Registry data by Kim and colleagues [6] quantified the incidence of skin breakdowns reported between 2009 and 2012 among Registry participants at 26%; on their most recent visit, 19% of patients > 2 years old had skin breakdown.

A Working Group was convened. The work of the group included a review of the skin breakdown prevention literature and clinical practices of Registry clinics related to skin breakdown. Most clinics did not have specific tools to use to educate patients and families about the care of insensate skin.

In collaboration with the SBA, a Skin Breakdown Prevention Care Bundle was created. A Care Bundle is a set of interventions that, when used together, significantly improve patient outcomes. The Skin Breakdown Prevention Bundle includes a skin assessment tool, skin breakdown risk assessment tool, educational brochures for three age groups, and a poster for educating the patient and family. The content has been translated into Spanish.

The Skin Breakdown Prevention Care Bundle also includes an online training presentation for use of the bundle and a staff training manual that includes goals and activities to prevent skin breakdown relative to each risk factor. The Skin Breakdown Prevention Bundle was

implemented in 10 Registry clinics in 2016. Monitoring the use of the bundle is done through the registry. Clinics are informed quarterly of the proportion of patients seen who received the prevention information. Skin breakdown incidence by clinic is also reported. A visual reminder for the patient and family, as well as an electronic application that provides information based on the patient's particular risk for skin breakdown, will be developed.

6. Conclusion

The NSBPR was implemented to improve the care and health outcomes of people living with spina bifida and to build a foundation for research. The activities of the Registry have led to collaboration among clinics. The variation in practice and outcomes is shared openly among participating clinics to provide opportunity for discussion about the causes of variation and the opportunities for improvement. Research using Registry data has begun to describe practice and outcome variation in spina bifida clinics. Future exploration of clinic processes and practices may continue to identify best practices, such as maintaining skin integrity, which, if adopted, could improve the health outcomes for all people living with spina bifida.

The reach and effectiveness of the NSBPR is enhanced by the activities of the SBA and their work to develop and implement the infrastructure for Spina Bifida Clinical Care Monitoring and Tracking. This system will monitor, track and evaluate patterns of care provided in spina bifida clinics with the goal of improving and standardizing care through evidence-based effectiveness analysis. This will result in a system that coordinates communication between clinics and local chapters, provides input into research priorities for the NSBPR, and educates and informs relevant parties: healthcare systems, healthcare professionals, people with spina bifida and their families, and payers, with the goal of supporting the adoption of best practices identified via the NSBPR and associated research and quality improvement projects.

Acknowledgments

The findings and conclusions in this presentation are those of the author and do not necessarily represent the official position of the Centers for Disease Control and Prevention.

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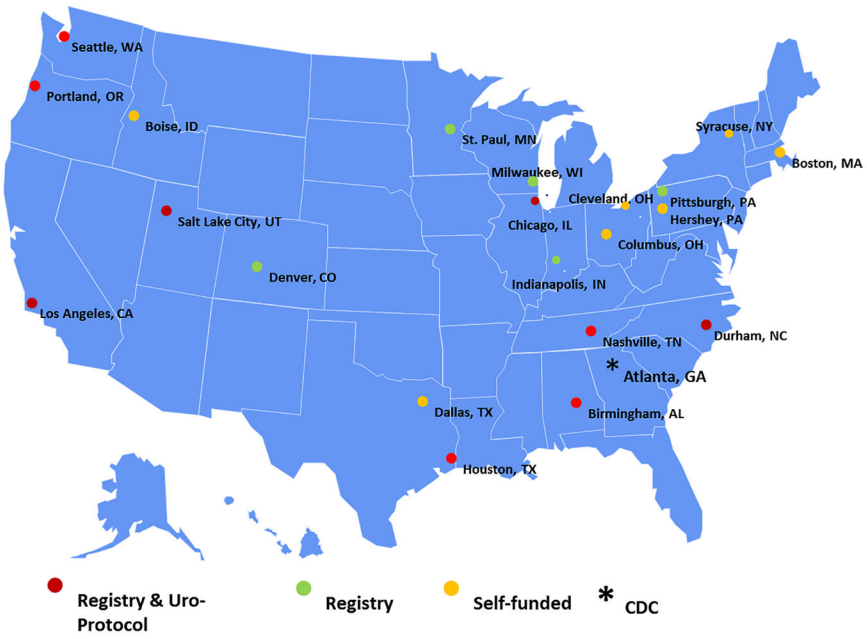

Fig. 1.

Geographic distribution of multidisciplinary clinics participating in the National Spina Bifida Patient Registry, 2017. Clinics participating in the registry and in the assessment of a urology protocol for newborns and young children, are indicated on this map by a red dot; clinics participating in the registry only are indicated by a green dot; and clinics that are participating in the registry and are self-funded are indicated by a gold dot.

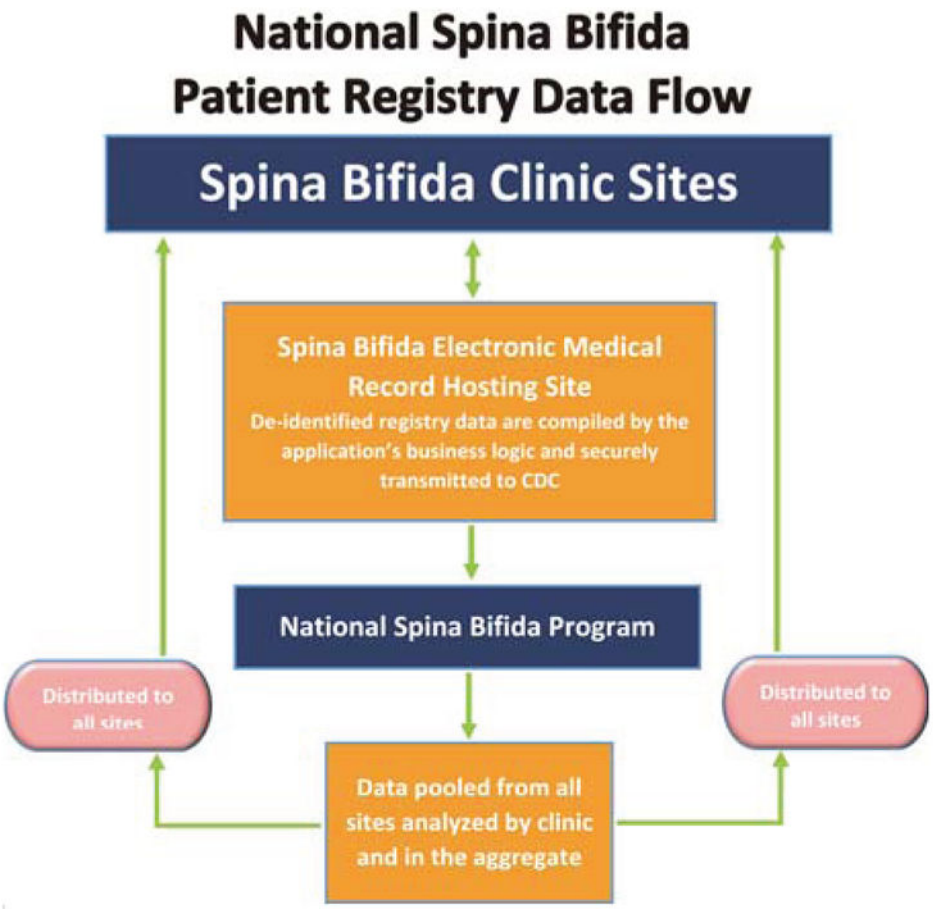

Fig. 2.
National spina bifida patient registry data flow.

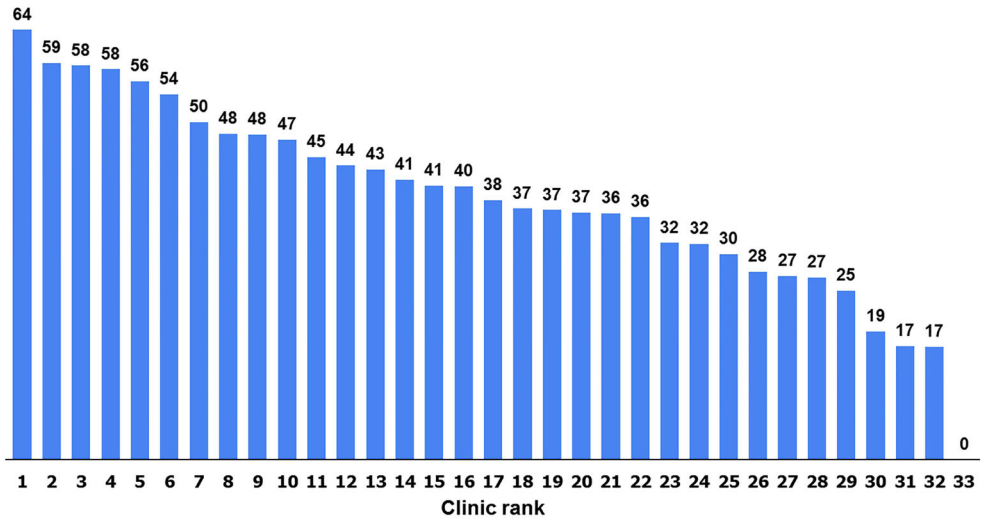

Fig. 3.

Percent of myelomeningocele patients aged 5+ years with urinary continence across clinics participating in the NSBPR (2009–2016) (N = 4,932).

Table 1

Characteristics of registry patients with spina bifida (n = 7,924)

Characteristic	Percent (%)
Sex *	
Female	52
Ethnicity *	
Non-Hispanic white	63
Hispanic or Latino	22
Black	7
Other	8
Health insurance coverage *	
Some private	48
Public	46
History of shunt placement	
Yes	77
Urinary continence **	
Yes	40–52
Bowel continence **	
Yes	47–69
Prenatal back closure	
Yes	4

* At the last reported annual visit.

** Among those five years old or older.

Table 2

Work/School status of patients with spina bifida, 22 years old or older (n = 1,183)

Vocational status	Percent (%)
Employed, full- or part-time	35.7
Permanently disabled/not employed	34.3
Seeking work	13.5
Child/student	7.3
Volunteer	4.6
Homemaker	4.5
Retired	0.1

Table 3

Surgical history of patients with myelomeningocele (n = 6,214)

Surgeries	Percent (%)
Shunt placement	77
Chiari decompression	9
Bladder augmentation	14
Antegrade continence enema	16

Table 4

Surgical history of patients with other eligible diagnoses (n = 1,1710)

Surgeries	Percent (%)
Colostomy	6
Shunt placement	5
Bladder augmentation	5